

# Takeda, National MPS Society, Luna, and Genetic Alliance Launch Community-Led Discovery Program for Hunter Syndrome

Families make the difference in accelerating research



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Dec 14, 2021, 10:00 ET

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SAN DIEGO, Dec. 14, 2021 /PRNewswire/ -- National MPS Society, Luna, and Genetic Alliance launched a digital drug discovery community in partnership with Takeda Pharmaceutical Company Limited ("Takeda") to advance the understanding of and develop therapeutic interventions for patients with Mucopolysaccharidosis type II (MPS II), also known as Hunter syndrome.

Sponsored by Takeda, the program leverages whole-genome sequencing to create a rich, longitudinal data stream that can be viewed across different lenses between industry and the patient community. The comprehensive study – inclusive of input from patients and families, patient advocates, and patient-centric data stewardship – is designed to ensure that the most relevant clinical and behavioral features and key endpoints are incorporated in the drug discovery process. Access to these data streams, including medical history and patient-generated health data, will form the basis for discovery and development. Genetic Alliance is providing the partners with engagement and regulatory expertise for this program.

"We are excited to work with Takeda in exactly our sweet spot. It is we who know these families and their affected children the best. They look to us for support and long-term solutions," said Terri L. Klein, CNPM, president and CEO of the National MPS Society. "We are very happy to be a

integral partner in this important study to engage and support MPS II patients and their families every step of the way."

MPS II is a rare lysosomal inborn error of metabolism caused by mutations in the iduronate 2-sulfatase (IDS) gene that affects every organ of the body. Current knowledge of the disease and limited access to deep longitudinal clinical data support the need to conduct whole genome sequencing to better understand disease natural history, disease heterogeneity, and the contribution of IDS mutations as well as genetic modifiers outside of IDS to disease presentation. More than 600 IDS disease-causing mutations have been implicated in MPSII. While some mutation types are typically associated with neuronopathic disease and cognitive impairment, single nucleotide mutations show variable association with other disease manifestations.

"For more than three decades, Genetic Alliance has created and deployed tools that put families in charge of their health, sharing their health data to help drive research," said Sharon Terry, president and CEO of Genetic Alliance. "The study framework allows for patients to participate entirely virtually, which reduces the burden on families. This allows greater participation from more diverse populations, expedites study recruitment, and amplifies the statistical power for discovery -- creating unique benefits for both participants and researchers."

"The National MPS Society and Genetic Alliance play a leading role in this program to ensure a family-centered study design -- one that is consistent with their needs, wants, and lifestyles," said Dawn Barry, president and Co-Founder of Luna. "Participants are asked to share what matters most to them about living with MPSII so that the most important endpoints are incorporated. The program also includes the return of data to participants, the opportunity to engage with a genetic counselor, and the ease of participating from their own homes."

Although the age of onset, disease severity, and rate of progression of the MPSII varies significantly, initial symptoms usually become apparent in children from two to four years of age. There is currently no cure for this condition which primarily affects 1 in 162,000 total live births, and almost exclusively males.

"This project exemplifies the interdependency between research and the patients and families who we hope will one day benefit from potential breakthroughs that result from the program," said Dan Curran, M.D., Head, Rare Genetics & Hematology Therapeutic Area Unit at Takeda. "We

have had the privilege of working with the National MPS Society and the global Hunter syndrome community for many years as part of our ongoing commitment to delivering novel therapies, disease education and support resources to those affected by this rare lysosomal storage disorder. We are proud to partner together to create more patient-centered discovery programs."

Visit National MPS Society program page to learn more.

## **About LunaPBC**

Founded in 2017, LunaPBC is a public benefit corporation headquartered in San Diego, California. The team, investors, and advisors are renowned in the patient-advocacy, health, and science fields. With participation from over 180 countries and communities advancing causes including disease-specific, public health, environmental, and emerging interests, Luna's tools and services empower these collectives to gather a wide range of data -- health records, lived experience, disease history, genomics, and more -- to advance research that addresses their unique health needs. Luna makes research representative of the real world and aligned with people's true goals by giving all participants a role from right where they are. For more information, visit [LunaDNA.com](https://LunaDNA.com).

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